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Webinar: Focus on the Eye



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Presentations from FDA and Leading Experts

May 2, 2012

The Webinar was held on May 2, 2012

- [Click here to watch and listen to a recording of the webinar.](#)

Slides for each presenter are also available by clicking on the links below.

Moderator: Ellen Feigal, MD, Senior Vice President of Research and Development, CIRM

Speakers:

- **Samuel Barone**, MD, [download slides] Medical Officer, Office of Cellular, Tissue and Gene Therapies (OCTGT), Center for Biologics Evaluation and Research (CBER), U.S. Food and Drug Administration (FDA)
- **Gary Rabin**, Chairman and CEO, **Matthew Vincent**, Ph.D., [download slides] Director of Business Development, and Edmund Mickunas, Vice President of Regulatory Affairs, Advanced Cell Technology (ACT)
- **Mark Humayun**, MD, Ph.D., [download slides] Professor of Ophthalmology, Biomedical Engineering and Cell and Neurobiology at the Keck School of Medicine of the University of Southern California (USC) as well as the associate director of research at the Doheny Retina Institute at USC

Topics to be covered:

- Presentation by FDA - overview of FDA regulations for Cellular and Gene Therapies for Retinal Disorders including combination products
- Examples of issues and challenges of taking an ophthalmic product through to an Investigational New Drug (IND) application
- Points to consider in clinical trial design for retinal disorders - immune monitoring and selecting appropriate endpoints
- Questions and Answers A written set of the questions and answers is posted here [pdf]

Background material:



- CIRM Macular Degeneration Fact Sheet
- List of awarded CIRM grants related to vision loss
- Funding opportunities
- CIRM: Strategic Partnership I Awards - RFA 12-05 Applications due June 26, 2012

- Department of Health and Human Resources, National Institute of Health, National Eye Institute: Stem Cell Approaches for Developing New Therapies for Ocular Diseases (R01) – RFA-EY-12-001 Applications Due July 2, 2012
- Research conferences and meetings related to vision loss
 - Louis J. Fox Center for Vision Restoration, 2012 Conference: Regenerative Medicine in Ophthalmology, May 11, 2012, University of Pittsburgh, Pennsylvania [Click here for more information]
 - 6th Sayer Vision Research lecture and NEI Symposium on Stem Cells, April 16, 2012. The intramural science program of the National Eye Institute, in collaboration with the NIH Center for Regenerative Medicine, is organizing its second symposium on Stem Cells, to be held on the NIH Campus in Bethesda on Monday, April 16, 2012. The symposium will focus on advances in stem cells research as they relate to ocular physiology and disease analysis. [Click here for more information]
 - FDA's Cellular, Tissue and Gene Therapies Advisory Committee Meeting Presentation: Cellular and Gene Therapies for Retinal Disorders, June 29, 2011 [Click here for more information]
- Stem cell therapeutic applications in ophthalmology

Table 3. Stem Cell/Cell Therapy in Ophthalmology - Ongoing & Completed Clinical Trial Details

Disease State	Clinical Trial	Sponsor	Clinical Sites	Status	Number Patients to be Studied	Number Studied to Date
Stargardt's Disease	NCT01345006	Advanced Cell Technology	<ul style="list-style-type: none"> • UCLA/Jules Stein - R* • Wills Eye Institute - R • Bascolm Palmer -R 	Phase I/II	12	6
	NCT01469832		<ul style="list-style-type: none"> • Moorfields -R • NHS Lothian (Scotland) - R 	Phase I/II	12	4
	NCT01625559	CHA Bio & Diostech (S. Korea)	CHA Bundang Med. Cntr. - NYR	Phase I	3	
Glaucoma Primary Open Angle	NCT01408472	Dr. Jeffrey Goldberg Univ. of Miami	Bascolm Palmer - NYR	Phase I	10	
Macular Telangiectasia (MacTel)	NCT01327911	Neurotech	<ul style="list-style-type: none"> • UCLA/Jules Stein - NYR • Retina Assoc. of Cleveland - NYR 	Phase I	7	

- Stem cell companies active in ophthalmology

Table 1. Stem Cell Companies Active in Ophthalmology

Company	Collaborator(s)	Cell Type	Applications
Advanced Cell Technology Inc. (ACT)	Oregon Health & Science University (OHSU) <ul style="list-style-type: none"> Moorfields Eye Hosp. UCLA/Jules Stein Wills Eye Hospital Aberdeen Royal Infirm. Bascom Palmer 	hESCs	RPE cells for retinal diseases, including Dry AMD NCT01344993 and Stargardt's NCT01345006 NCT01469832
AstraZeneca	Univ. College London (UCL)	hESCs	Diabetic Retinopathy
Centecor/J&J w/ iScience Interventional	<ul style="list-style-type: none"> Retina Institute of CA Wills Eye Hospital 	Adult SCs (CNT0 2746 from umbilical cord fluid)	RPE cells for GA (atrophic dry AMD) NCT01226628
Cell Cure Neurosciences	TEVA	hESCs	RPE cells for dry AMD
CellSeed France SARL	FGK Clinical Reserach GmbH <ul style="list-style-type: none"> Universitätsklinikum, Erlangen, Germany 	oral mucosa epithelial cells	CAOMECS* for Corneal tissue repair in those with limbal cell deficiencies NCT01489501
Fundación Clinic per a la Recerca Biomedica (Spain)	<ul style="list-style-type: none"> Hospital Clinic Barcelona Instituto Univ. Barraquer Instituto de Microcirugía Ocular 	sclerocorneal progenitor cells from the limbus	Replacement of damaged corneal cells NCT01470573
General Hosp of the Chinese People's Armed Police Force (China)		unknown	Optic Nerve Atrophy ChiCTR-TNRC-11001491
International Stem Cell Corp. (ISCO)	CytoCor - <ul style="list-style-type: none"> Absorption Systems - US Sankara Nethralaya - India Automation Partnership - UK CytoRet - UC Irvine	hpSCs	Corneal tissue for transplantation into degenerated corneas
		hpSCs	RPE cells and layered retinal structures for AMD

- Ongoing & Completed Clinical Trial Details for Stem Cell/ Cell Therapy in Ophthalmology

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	NCT01469832		<ul style="list-style-type: none"> Moorfields -R NHS Lothian (Scotland) - R 	Phase IIa	4	1
	NCT01625559	CHA Bio & Diostech (S. Korea)	CHA Bundang Med. Cntr. - NYR	Phase I/II	12	7
	NCT01736059	Univ. Cal. Davis	Univ. Cal. Davis - R	Phase I	3	1
Glaucoma Primary Open Angle	NCT01408472	Dr. Jeffrey Goldberg Univ. of Miami	Bascolm Palmer - NYR	Phase I	2-3	
Macular Telangectasia (MacTel)	NCT01327911	Neurotech	<ul style="list-style-type: none"> UCLA/Jules Stein - NYR Retina Assoc. of Cleveland - NYR 	Phase I	10	7

- *charts supplied by **Irv Arons** and are current as of June 2013*

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